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Decision-making by healthcare payers

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Abstract Healthcare payers are faced with the need to allocate finite resources to maximize population health. To assist in decision-making, healthcare payers are increasingly using health outcomes information and economic analyses. Healthcare payers are often under pressure to make early decisions (around the time of product launch), when the evidence available is imperfect. They must also consider the equitable distribution of resources between therapeutic areas. Tools to help healthcare payers reach transparent and objective decisions include cost-utility analysis and decision modelling. In practice, healthcare payers in different countries (for example, Ireland, France and Canada) vary in the approaches taken to reimbursement and formulary listing decisions. The key to decision-making among healthcare payers is the provision of appropriate evidence, comparing any new treatment approach to current best practice, in situations corresponding to real life. When data assumptions have to be made, these should be clearly stated with consideration of the impact of varying the assumptions. The impact on budgets should also be considered.

Keywords: chronic obstructive pulmonary disease; healthcare payers; decision models; QALYs; cost-effectiveness; utility

INTRODUCTION

Healthcare systems in the developed world are facing increasing demands, arising from aging populations, technological advances and increased patient expectations. As a result, governments and healthcare payers are faced with the need to allocate limited resources for maximum benefit (1,2). To assist in decision-making, healthcare payers are increasingly using health outcomes information and economic analyses. This paper aims to describe some of the difficulties faced by healthcare decision-makers and some tools being used to overcome these problems, and briefly summarizes examples of the way healthcare payers make decisions in different healthcare systems.

DIFFICULTIES IN HEALTHCARE PAYER DECISION-MAKING

Healthcare payers are charged with the responsibility to reach a decision on whether or not to fund particular therapeutic approaches, based on balancing the costs and benefits of the therapy. If it is clear that when a treatment costs more than alternatives while delivering fewer benefits (and/or more harms), the decision not to fund the treatment can be easily made. There is also a minority of cases where the therapy can be clearly shown to cost less than alternative approaches while generating more benefits – in these cases, it could be expected that healthcare payers should also have few problems with making the decision to fund the treatment.

More commonly, healthcare payers have to decide whether or not to recommend treatments that either cost less and deliver fewer benefits than alternatives, or cost more but deliver greater benefits than previous therapies. In such cases, wider issues of budget impact must also be considered. Decisions have to be taken on whether the extra resources required can be obtained by cutting back elsewhere, so that total benefits from a relatively fixed budget can be increased, or if additional funds can be found.

A further difficulty faced by decision makers considering the introduction of a product with clear clinical benefits over alternative treatments is the potential impact on prescriber behaviour and patient expectations. Even if the product represents good value for money, increased demand may result in considerable impact on budgets. For example, when the highly selective serotonin reuptake inhibitors were developed for the treatment of depression, they were shown to be at least as cost-effective as traditional treatment with tricyclic antidepressants. The expectation was that there

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would not be a dramatic increase in prescribing costs. Yet greater demand for effective treatments that had relatively few side-effects led to an exponential increase in prescribing costs (3).

Healthcare payers are under pressure to make an early decision on funding status to ensure that new treatments become available promptly. Partly as a consequence of this time pressure, the evidence on which the decision must be based will often be imperfect. For example, there may be problems with the quality of the evidence available, which, in the case of pharmaceuticals, commonly comes from randomized controlled clinical trials that, early in product development, tend to be restrictive in patient population and of small scale. Furthermore, the focus of the evidence may be geared towards the need for regulatory approval, failing to address the specific needs of healthcare payers (4). Common difficulties with the evidence, from the healthcare payer perspective, include inappropriate comparisons, use of intermediate outcomes, inadequate duration of follow-up and lack of data on resource use and economic impact. These problems were highlighted in a review of industry submissions to the agency considering reimbursement in Australia (5). Of 326 submissions between 1994 and 1997, 218 (67%) had a total of 249 significant problems, with the majority of these (62%) related to estimates of comparative efficacy. In a review of 21 submissions to a Canadian reimbursement agency, only five submissions were judged to have complied with the guidance given on evidence required (6).

Early technology assessment carries the risk that inappropriate decisions will be made – a treatment that is truly clinically ineffective or cost-ineffective could be recommended for use in practice (with subsequent difficulties in removing it from the armamentarium), or a treatment that is truly clinically effective or cost-effective could fail to be recommended (with potentially adverse consequences for patients). Furthermore, in addition to costs and benefits, healthcare payers must take into account other issues, such as impact on specific subgroups of patients. There is also a need to balance equitable resource use across different disease or therapeutic areas. And underlying all the difficulties facing healthcare payers is the need to ensure that decisions are taken in a fair and transparent manner.

In seeking to address the difficulties that healthcare payers face when making decisions, a number of tools have been developed. Healthcare payers are making increasing use of cost-utility analysis, to assist in the efficient allocation of resources across therapeutic areas. To address the difficulties inherent in early technology assessment, most decision-making bodies are willing to recommend the collection of more evidence, particularly in subgroups of patients who are most likely to benefit from treatment, with revisiting of the funding decision in the light of the new evidence. Healthcare payers are also seeking to address shortcomings in evidence by using modelling to synthesize data from a range of sources including explicit judgements by experts. Such an approach also makes explicit assumptions, thereby facilitating transparency of decision-making. The tools of cost-utility analysis and decision-modelling are considered in more detail below.

COST-UTILITY ANALYSIS

Healthcare payers deciding on the efficient allocation of resources need to be able to consider both improvements in quality of life gained by a treatment, and the impact on duration of life, as well as the cost required to achieve these gains. This requires considerable synthesis of information into a single scale, so that direct comparisons can be made across therapeutic areas. The scale often used by healthcare payers is the cost per quality-adjusted life year (QALY) gained. The following is a brief description of the calculation of cost per QALY gained.

Measuring utility

The first stage in calculating cost per QALY is to assess the quality of different health states. This is expressed in terms of the utility, that is, the desirability of different health states. Utility is measured by asking patients or a wider population to state their views on different health states. One method of collecting these views that is gaining increasing favour is the use of questionnaires (such as the Health Utilities Index (7) and the EQ-5D (8)) that ask about different attributes of health. The attributes assessed by the EQ-5D are shown in Table 1. A total of 245 different health states can be defined using the EQ-5D (i.e. 3^5 for the three possible answers in five attributes, plus the health states of unconscious and dead). Each of these health states is then assigned a value on a scale of 0 (death) to 1 (perfect health), obtained by asking the opinion of a wide sample of the general public. In the U.K., the sample consisted of 3395 members of the public (9). For example, the mean value assigned to the health state consisting of some problems in walking about, but no problems with self-care or performing usual activities and no pain/discomfort or anxiety/depression was 0·85, while a mean value of 0·52 was assigned to the health state consisting of some problems with walking about, self-care and usual activities, with moderate pain/discomfort and moderate anxiety/depression.

Quality-adjusted life years

Once the utility value placed on a patient's health is obtained, the time spent at each utility value can be assessed, as shown diagrammatically in Fig. 1, so that the

Table 1. Attributes of health assessed in the EQ-5D (8)

Attribute	Range of functioning
Mobility	I have no problems in walking about I have some problems in walking about I am confined to bed
Self-care	I have no problems with self-care I have some problems washing or dressing myself I am unable to wash or dress myself
Usual activities (e.g. work, study, housework, family or leisure activities)	I have no problems with performing my usual activities I have some problems with performing my usual activities I am unable to perform my usual activities
Pain/discomfort	I have no pain/discomfort I have moderate pain or discomfort I have extreme pain or discomfort
Anxiety/depression	I am not anxious or depressed I am moderately anxious or depressed I am extremely anxious or depressed

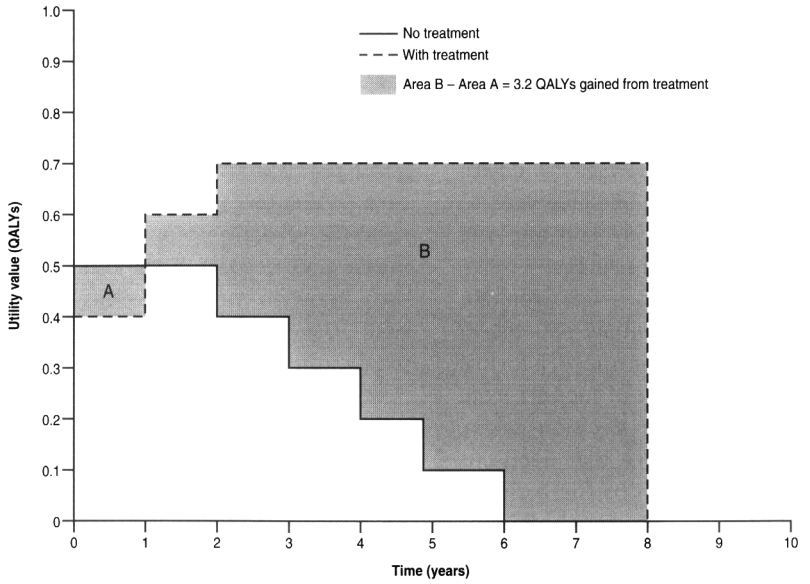

FIGURE 1. Calculation of quality-adjusted life-years with and without a hypothetical treatment.

number of QALYs can be calculated by multiplying each utility value by the time spent at that value, and summing all the individual elements over the time period. This figure also shows the time at each utility value if a hypothetical treatment is given. The number of QALYs gained by the treatment is the difference between the QALYs with and without the therapy.

Cost per QALY

Alongside assessments of QALYs, costs can be taken into account. These usually include the cost of the treatment itself and the alternative form of management, together with other relevant healthcare costs, such as the cost of consultations, management of any adverse events from treatment and costs arising from treatment failure.

Using cost-utility analysis in decision-making

Once a cost-utility analysis has been carried out, healthcare payers can compare the value of the therapy, in terms of its extra cost per additional QALY, with treatments in other disease areas. This allows a threshold

to be used, either implicitly or explicitly, below which treatments may be funded. In the U.K., for example, although no explicitly stated threshold for cost per QALY of treatments deemed economically acceptable has been published, analysis of 19 recent decisions taken by the National Institute for Clinical Excellence (NICE) showed that only around half cited a cost per QALY (10). Of these, positive recommendations were given to all but one treatment with a cost per QALY below £30 000, partly achieved by restricting use to those patients experiencing greatest benefits from treatment. Treatment with riluzole for a subgroup of patients with motor neuron disease was recommended, despite the cost per QALY of £34 000-£44 000, because of the severity of impairment of patients and short survival time. Other treatments with a high cost per QALY (£40 000-£90 000) were not recommended. In the U.S.A., an implicit threshold of $50 000 per QALY has been observed.

DECISION MODELLING

Medical practice involves making hundreds of decisions each day. Evidence comparing alternative treatment approaches obtained from trials, systematic reviews and meta-analyses, together with hidden values and assumptions derived from their training and experience, allow clinicians to arrive empirically and intuitively at decisions, which, hopefully, are appropriate in the majority of cases. Developments, such as the use of evidence to devise treatment protocols and management guidelines, seek to reduce inappropriate decision-making by collating and weighting information from a variety of sources.

In the more recent field of healthcare management, such guidelines that draw together multiple sources of evidence are not always available. Furthermore, informal synthesis of information can lead to lack of support from key stakeholders and distrust of the decision taken. Consequently, there is a need to ensure that decisions having potentially far-reaching implications for patients, healthcare professionals, manufacturers and politicians alike (such as a recommendation to use or not use a particular medication) should be taken explicitly and on the basis of a clear and rational framework. Such an explicit framework is provided by decision modelling, which also attempts to address the difficulties inherent in making decisions under conditions of uncertainty and where complete data is not available.

What is a decision model?

A decision model is a mathematical prediction of health-related events, each of which are linked to costs and health outcomes. It usually compares mutually exclusive interventions for a specific patient group, synthesizing data from various sources. Where data is incomplete, explicit assumptions are made, and the impact of changes in these assumptions can be investigated so that assessment of uncertainty can be made. Decision models used by healthcare payers synthesize economic and clinical outcomes to answer appropriate questions (Fig. 2). Such models are not alternatives to clinical trial data – rather they are a tool to make explicit the factors that are being considered in a decision, rather than allowing these factors to be considered implicitly (Fig. 3). This approach allows discussion of the unknown parameters and agreement on the assumptions to make, before seeing the associated outcomes, thereby aiding discussion and debate among decision-makers. Such an approach gives an objective framework to a decision.

Recently, the International Society for Pharmaceutical Outcomes Research (ISPOR) published the principles of good practice for decision analytic modelling in health care evaluation (11). These principles emphasized the need for validation of models internally (to calibrate the model and ensure there are no bugs hampering their use), between models (to ensure results are consistent with other approaches) and externally (to make predictions). Methodolgical guidelines on decision modelling were also published following a consensus conference on economic modelling in health technology assessment, held in England in 1999 (12).

Using decision modelling

Decision models have been used to assess cost-effectiveness, without restriction to those issues that are

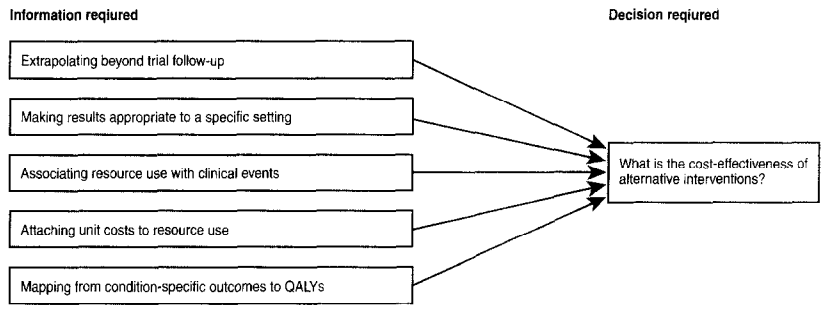

FIGURE 2. Synthesis of information in respiratory disease required in healthcare payer.

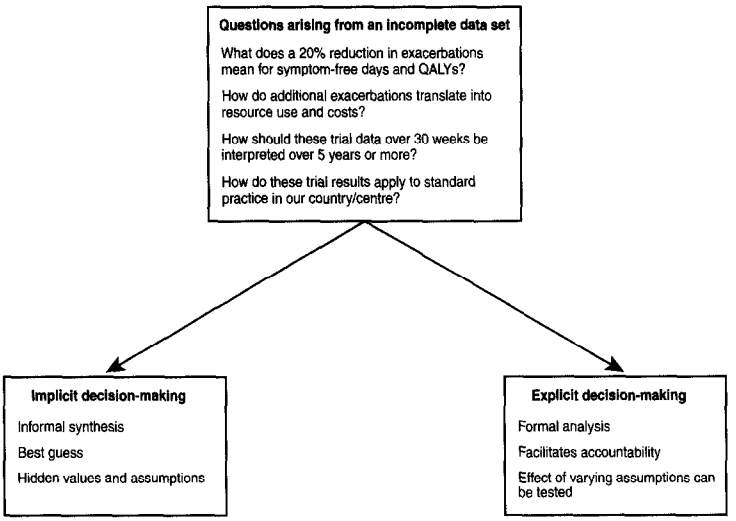

FIGURE 3. Implicit and explicit decision-making.

measured in clinical trials, so that all ways that patients could be managed are included. For example, in the field of respiratory care, a decision model has been developed to assess the cost-effectiveness of alternative asthma management strategies (13). This model is based on the probability of moving between different health states, each of which is associated with a specific cost. The transition probabilities are determined, where possible, from clinical trials and other evidence – where evidence is lacking, explicit assumptions are made that can be altered to test the impact.

The overall impact of a treatment on transition through the health states over a specified time period can be tested, with the results presented on a cost-effectiveness plane (Fig. 4). Estimates are repeated to reflect the uncertainty in parameters, with the clustering of points giving an indication of confidence in the findings. For example, the model of asthma management strategies showed that Seretide was comparable in cost to the comparative management approach, while resulting in improved health outcomes (Fig. 5) (13). Results can also be presented as a cost-effectiveness acceptability curve, whereby the probability of a treatment being cost-effective is plotted against varying amounts that the decision maker may be willing to pay for an additional unit of health outcome.

In chronic obstructive pulmonary disease (COPD), work is underway to develop appropriate models to aid decision-making, though this is hampered by the lack of agreement on a clear way of defining the natural history of the condition.

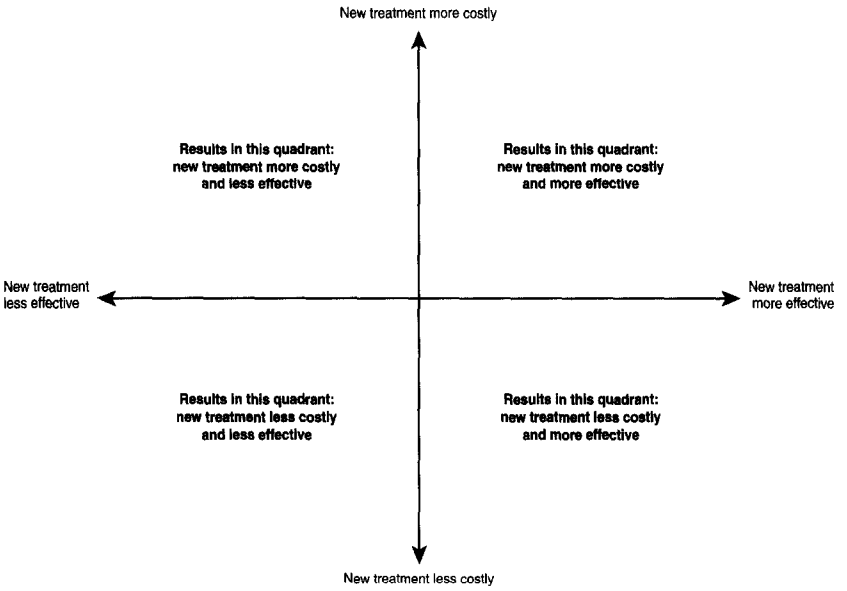

FIGURE 4. Cost-effectiveness plane, used to present the results of an analysis comparing a new and old treatment.

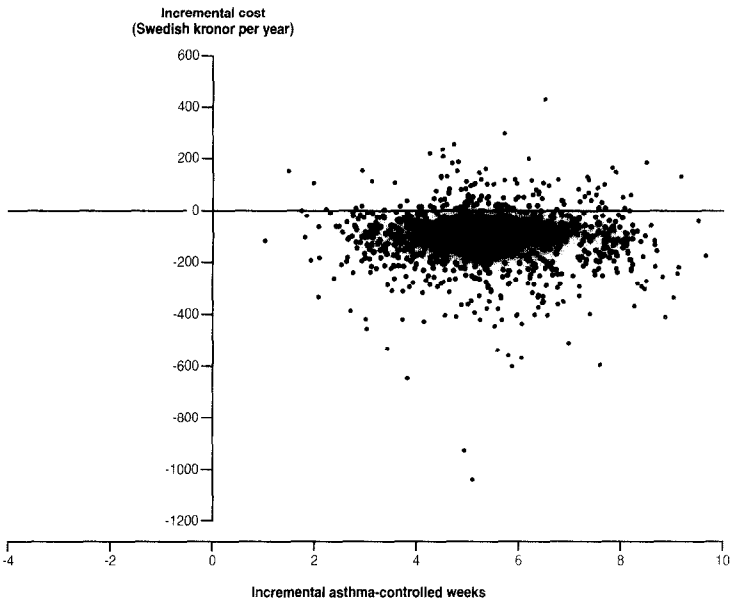

FIGURE 5. A decision model of asthma management showed that treatment with Seretide (salmeterol/fluticasone propionate) was similar in cost to comparative therapy (formoterol/budesonide) but more effective (13).

DECISION-MAKING IN PRACTICE

Having discussed some of the difficulties faced by healthcare payers when making decisions about the equitable distribution of resources between therapies and management approaches, and considered two of the tools being used, this section of the paper briefly summarizes healthcare payer decision-making in practice, focussing on reimbursement and formulary listing in Ireland, France and Canada.

Drug reimbursement in Ireland

In Ireland, as in other developed countries, there has been a rapid increase in public expenditure on pharmaceuticals in recent years – rising from €227 million in 1993 to €531 million in 2000 (14,15). Drugs having the highest impact on the budget (that is, acquisition cost multiplied by the volume) include a number of products used for the treatment of respiratory conditions. Overall, 11% of pharmaceutical expenditure is for respiratory products (14). There are 11 different schemes covering pharmaceutical reimbursement in Ireland, with the majority of patients (57·3%) registered as being eligible to benefit under one of the General Medical Services schemes (16,17). Although prescriptions are free for those on low incomes and for the elderly, there is co-payment for other patients. Patients with some chronic diseases (though not asthma or COPD) receive free prescriptions. Pricing of pharmaceutical products varies with the reimbursement scheme, but generally includes the manufacturers price with the addition of a wholesale margin (17·47%) and a dispensing fee (€2·31–2·65). In some schemes, a retail margin (50%) is also added. For the most expensive medications, the retail margin and dispensing fees are replaced with a monthly patient care fee of €43·93 (16).

In an attempt to control expenditure, an indicative drug target scheme has been introduced, which aims to reduce the level of prescribing without adversely affecting the quality of care by promoting therapeutic and generic substitution, and cost-effective prescribing, while discouraging prescribing of drugs with limited clinical efficacy (18). Each general practitioner is given a target for expenditure, based on the age and gender of the practice population, and a proportion of any savings made can be retained by the practice for service development. Part of the budget is allocated to expenditure on budget neutral products (e.g. statins, nicotine replacement therapy). There are also some restrictions on volume of treatments (e.g. sildenafil) that can be prescribed, while other products are restricted to use by consultants.

In Ireland, the price of new products is linked to the lesser of the U.K. price and the average price of the same product in a basket of European countries (Denmark, France, Germany, Netherlands and U.K.), and once the price is agreed, it remains fixed and is only very rarely renegotiated (19). There is no direct barrier to reimbursement in Ireland. However, since August 1997, the Department of Health has reserved the right to seek cost-benefit studies for any new chemical entity introduced to Ireland (20). Such economic analyses are evaluated by the National Pharmacoeconomic Centre, which also conducts research into high cost therapeutic areas and is involved in education to advance the understanding of pharmacoeconomics in Ireland (21).

The centre has developed health technology assessment guidelines, similar to those in use by the National Institute for Clinical Excellence in England and Wales (22). The guidelines are user-friendly and flexible, providing an opportunity for industry to present and discuss the economic data available. A pragmatic approach is taken to health technology assessment, with decision-makers accepting that studies may not have been conducted in Ireland – there is concern that insisting on national studies could discourage manufacturers from introducing innovative products into the small Irish market. Although pharmacoeconomic evaluation has not taken a major role in decisions about reimbursement and formulary listing in Ireland, this is likely to change in the future.

Reimbursement in France

The reimbursement system in France changed 2 years ago. Now, there has to be explicit demonstration of the benefits of new drugs to justify reimbursement (2). Reimbursement of products is at varying rates – for example, drugs for HIV infection and cancer receive 100% reimbursement, while those for asthma are reimbursed at 65% and those showing only limited benefits are reimbursed at 35%. However, the majority of patients have health insurance to cover co-payment of drugs, so patients are not directly affected by the financial consequences of reimbursement decisions.

Formulary access in Ontario, Canada

In Canada, the provincial government of Ontario spends approximately Can$1·5 billion each year on pharmaceutical services for the elderly and for patients receiving social assistance (in total, amounting to 35% of the Ontario population). The provincial government has a responsibility to ensure that the budget is spent as efficiently as possible. When a pharmaceutical company wishes to launch a new product in Canada, they must first receive federal regulatory approval, before being assessed by the Patented Medicines Price Review Board. The latter sets the maximum Canadian price permitted, based on a median price derived from the U.S.A. and a basket of European countries.

The provincial drugs and therapeutics committee advises the provincial government whether or not it should pay for the drug for the beneficiaries of the province's drug programme. The committee consists of a chair and 11 members, each of whom can serve a maximum of 6 years, consecutively. Former members often serve as consultants to the committee. Manufacturers prepare submissions of clinical and economic data, for review by the committee. The key clinical evidence considered by the committee are the outcomes achieved with the therapy, with laboratory measures (e.g. FEV_1) having least influence, and progressively more influence from signs and symptoms, health-related quality of life (QALY) and survival. In ideal circumstances, at the top of the hierarchy of evidence is the impact on quality adjusted life years, as this measure captures both quality and quantity of life. However, this can be undermined if the committee is unable to trust the methods used to calculate the QALYs. Costs are considered from the perspective of the drug budget, the healthcare system (i.e. the Ministry of Health) and society. However, in reality, the societal perspective is given less weight as the recommendations are intended for use by the provincial Minister of Health. In addition to the efficacy and safety outcomes, and relevant costs and savings, the comparison used in the evidence has considerable impact on the recommended decision. Any new treatment must be compared with the therapy it is likely to replace. If not, reimbursement is unlikely to be considered until such evidence is available. Utilization potential is also important – that is, how the product is likely to be used in practice. The committee will then decide to give full listing on the provincial formulary, restricted listing or exclude the product from the formulary. In the last decade, there has been a reduction in the proportion of products given full listing, while many products have received listing for limited circumstances only. This trend is expected to continue in the future.

WHY CAN DEMONSTRATION OF GOOD VALUE FAIL TO INFLUENCE HEALTHCARE PAYERS?

In all the healthcare systems considered, as elsewhere in the developed world, the presentation of health outcomes information and pharmacoeconomic analyses demonstrating the value of a new product does not guarantee decision-making in favour of the product. There are a number of possible reasons.

- The wrong comparator has been used – possibly because trials have been designed for regulatory approval, involving comparison with placebo rather than customary care.
- Evidence is not representative of real use – as discussed in the previous paper by Mapel & Pearson, clinical trials may fail to include the populations of interest to healthcare payers, restricting the value of the data.
- Lack of precision – if there are too many assumptions, or these are not explicitly declared and able to be varied, decision-makers can lose confidence in the findings.
- Cost impact and lack of affordability – interestingly, this is probably not a common reason for rejection as the general aim of healthcare payers is not usually to reduce the budget, rather it is to ensure that the budget is spent wisely.

- Formulary access may be restricted to prevent physicians from prescribing the drug in circumstances where the value of the product is low.

CONCLUSIONS

In conclusion, healthcare payers are faced with the need to allocate finite resources in order to maximize population health. To assist in decision-making, healthcare payers are increasingly using health outcomes information and economic analyses. However, there is pressure to decide on funding status before or as soon as the product is marketed, though this may mean that there is limited evidence on which to base the decision. Furthermore, healthcare payers have to balance the needs of all therapeutic areas. To assist decision-making, tools that have been developed include cost-utility analysis, which considers costs, quality of life and quantity of life (QALYs), and decision modelling. Decision-makers may also use cost-effectiveness analysis to focus the availability of drugs to patient subgroups or patient indications where the cost-effectiveness is shown to be most favourable. The key to decision-making among healthcare payers is the provision of appropriate evidence, comparing any new treatment approach to current standard practice, in situations corresponding to real life. When assumptions have to be made, these should be clearly stated with consideration of the impact of varying the assumptions. The impact on budgets should also be considered.

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